Lambert-Eaton Myasthenic Syndrome (LEMS) Registry (LEMS-01)

First published: 21/07/2014

Last updated: 29/01/2021

Study Finalised

Administrative details

PURI

https://redirect.ema.europa.eu/resource/39278

EU PAS number

EUPAS6106

Study ID

39278

DARWIN EU® study

No

Study countries

- ☐ France
- ☐ Germany
- ☐ Italy

☐ Spain

☐ United Kingdom

Study description

Lambert-Eaton Myasthenic Syndrome (LEMS) is a rare autoimmune disorder that affects voltage-gated calcium channels on the pre-synaptic membrane of the nerve-muscle (neuromuscular) junction. LEMS is estimated to affect 1 in 100,000 people in the European Community and is clinically characterized by weakness and frequent fatigue (mainly of the legs and trunk), and is associated with autonomic dysfunction (e.g. impotence, dry mouth, constipation). The onset of symptoms is usually gradual and insidious. The age at onset is typically ≥ 40 years in patients with cancer and between 20 and 50 years in those without. Slightly more men than women are affected. LEMS has also been reported in children, sometimes associated with neuroblastoma, but is an extremely rare condition in this patient group. In January 2010 the EU approved the Marketing Authorisation of 3, 4-DAP Phosphate (Amifamdripine, Firdapse®) for the treatment of LEMS. Firdapse is the only approved 3, 4-DAP compound for the treatment of LEMS. The purpose of the LEMS registry is to collect additional data on the long term safety and efficacy of Firdapse for patients who have been prescribed Firdapse by their treating physician. This registry will also increase knowledge about the course of disease in patients with LEMS by evaluating neurological and muscular function. The LEMS registry is a voluntary multi-centre, multinational, observational program for patients with LEMS disease, intended to track the routine clinical outcomes of patients with LEMS over time. As per condition of the EU Marketing Authorisation, the registry will also track the use of treatment for LEMS including drugs other than Firdapse. The data collected by the registry are intended to enable better characterisation of the natural history of LEMS.All patients with a confirmed diagnosis of LEMS disease may be eligible to participate in this program. No experimental treatments or assessments are involved in this program.

Study status

Finalised

Research institutions and networks

Institutions

BioMarin Pharmaceuticals

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Institution

Multiple centres: 30 centres are involved in the study

Contact details

Study institution contact

Program Director

Study contact

medinfoeu@BMRN.COM

Primary lead investigator

Program Director

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 01/10/2009

Actual: 09/01/2014

Study start date
Planned: 31/03/2010

Actual: 25/05/2010

Date of final study report
Planned: 28/06/2020

Actual: 21/02/2020

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin Clinical Limted

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Effectiveness study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

To obtain observational safety data (identification, frequency and severity) on patients diagnosed with LEMS. To monitor for safety signals in patients treated with Firdapse including long-term treatment.To evaluate the outcome of pregnancies in patients treated with Firdapse.To gather the same observational information from patients with LEMS not treated with Firdapse...

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational

Study drug and medical condition

Name of medicine

FIRDAPSE

Medical condition to be studied

Myasthenic syndrome

Population studied

Short description of the study population

Patients diagnosed with Lambert-Eaton Myasthenic Syndrome (LEMS).

Age groups

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Special population of interest

Other

Special population of interest, other

Lambert-Eaton Myasthenic Syndrome patients

Estimated number of subjects

105

Study design details

Data analysis plan

Registry data will be analyzed as per the program’s (SAP) and will be reported periodically to the European Medicines Agency (EMA) as per post marketing commitment. Longitudinal prospective and retrospective data may be collected. Demographic and baseline characteristics will be summarized. Frequencies will be presented for the categorical variables (e.g. sex and ethnicity), and descriptive statistics will be presented for continuous variables (e.g. height, weight, and age).Periodical descriptive statistical reports and final statistical analysis may also include:Patient accrual and follow-upExposure to Firdapse (dose, duration)Clinical responsePregnancy outcomes as appropriateAdverse Events (AE) including Serious AEs.AEs will be recorded as available.

Data management

Data sources

Data sources (types)

Disease registry

Electronic healthcare records (EHR)

Other

Data sources (types), other

Prospective patient-based data collection, Prescription event monitoring

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No